News Release

September 18, 2020
JCR Pharmaceuticals Co., Ltd.

Translation

JCR Receives MHLW Orphan Drug Designation for JR-141 (Pabinafusp Alfa) for Hunter Syndrome

JCR Pharmaceuticals Co., Ltd. (TSE: 4552; Chairman and President: Shin Ashida; "JCR") announced today that it has received orphan drug designation for JR-141 (Pabinafusp Alfa) for mucopolysaccharidosis II (Hunter syndrome) from the Ministry of Health, Labour and Welfare (MHLW) of Japan. JR-141 is a blood-brain-barrier (BBB)-penetrating recombinant iduronate-2-sulfatase product candidate for the treatment of patients with mucopolysaccharidosis II (Hunter syndrome), to which J-Brain Cargo®, JCR's proprietary BBB technology, is applied. JR-141 has received orphan drug designation in the United States in October 2018 and in Europe in February 2019; also, JCR plans to file a marketing authorization application for JR-141 in Japan in September 2020.

Mucopolysaccharidosis II (Hunter syndrome) is an X-linked recessive lysosomal storage disorder (LSD) caused by a deficiency of iduronate-2-sulfatase, an enzyme that breaks down glycosaminoglycans (mucopolysaccharides) in the body. The number of patients with Hunter syndrome in Japan is estimated at approximately 250 (JCR research). Hunter syndrome gives rise to a broad spectrum of symptoms. One major issue is that current enzyme replacement therapy cannot be expected to address central nervous system (CNS) disorders because the enzyme cannot exert effects on the brain due to its inability to penetrate the brain through BBB.

JR-141 is expected to suppress the onset or progression of CNS symptoms as a result of its ability to cross the BBB. In a Phase 3 trial conducted in Japan, the trial confirmed that the substrate accumulation concentrations in the cerebrospinal fluid of all trial participants decreased. The trial also generated meaningful results in terms of developmental assessment, a clinically significant assessment indicator. With regard to the safety profile, no adverse events of clinical concern were reported in connection with JR-141.

Following JR-141, JCR will harness its J-Brain Cargo® technology platform to develop a robust pipeline of innovative enzyme replacement therapy products for other LSDs. JCR, as a specialty pharma in the rare disease arena, will continue to proactively engage in research and development of treatment options for patients with rare diseases.

This designation is expected to have a minor impact on JCR's consolidated financial results for the year ending March 31, 2021.

About orphan drugs (MHLW)

Under the orphan drug designation system, MHLW takes special measures to support and promote research activities for the development of drugs for rare diseases. Drugs satisfying the following criteria may be designated as orphan drugs: The number of patients who may use the drug should be less than 50,000 in Japan. The drug should be indicated for the treatment of serious diseases. In addition, they must be drugs for which there are high medical needs, based on the following criteria: there is no appropriate alternative drug or treatment; or high efficacy or safety is expected compared with existing products. Moreover, there should be a theoretical rationale for the use of the product for the target disease, and the development plan should be appropriate.

[About JCR Pharmaceuticals]

JCR is a specialty pharma company engaged in the research, development, manufacturing and marketing of biopharmaceuticals and regenerative medicine with a focus on rare diseases. Its philosophy, “Contributing towards people’s healthcare through pharmaceutical products” drives JCR to create innovative pharmaceutical products as value-added treatment options for the under-served patient populations.

[Cautionary Statement Regarding Forward-Looking Statements]

This document contains forward-looking statements that are subject to known and unknown risks and uncertainties, many of which are outside our control. Forward-looking statements often contain words such as “believe,” “estimate,” “anticipate,” “intend,” “plan,” “will,” “would,” “target” and similar references to future periods. All forward-looking statements regarding our plans, outlook, strategy and future business, financial performance and financial condition are based on judgments derived from the information available to us at this time. Factors or events that could cause our actual results to be materially different from those expressed in our forward-looking statements include, but not limited to, a deterioration of economic conditions, a change in the legal or governmental system, a delay in launching a new product, impact on competitors’ pricing and product strategies, a decline in marketing capabilities relating to our products, manufacturing difficulties or delays, an infringement of our intellectual property rights , an adverse court decision in a significant lawsuit and regulatory actions.

This document involves information on pharmaceutical products (including those under development). However, it is not intended for advertising or providing medical advice. Furthermore, it is intended to provide information on our company and businesses and not to solicit investment in securities we issue.

Except as required by law, we assume no obligation to update these forward-looking statements publicly or to update the factors that could cause actual results to differ materially, even if new information becomes available in the future.

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